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Patient Centric Clinical Trials - The Future of Clinical Trials

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ABSTRACT

BACKGROUND:

The best quality evidence comes from randomized clinical trials (RCTs). The ultimate goal of any RCT is the betterment of the health of the patient. However; curiously, the patient is hardly a prominent stakeholder in most decisions related to such studies. This gap is gradually changing. Of late, researchers have started to realise the importance of including patient-centered outcome measures in clinical trial protocols - the so-called Patient-Centered Outcomes Research (PCOR). Even the regulators such as the USFDA are increasingly seeking patient-centered outcomes in RCT data for substantiating label claims. The concept of Patient-Centric Clinical Trials (PCCT) includes PCOR, and goes a step further by enhancing patient participation in the RCTs - ranging from the stage of protocol preparation and to making RCT data easily available to the participating patients, unlike in the present model, where patients come into picture only after the trial is started. Thus, even though patient-centered outcomes are included in the protocol, the patients themselves do not have a voice in selecting which outcomes are to be included. After RCT is completed, patients are hardly provided any information about its outcome. PCCT aims to bridge this gap, and more. PCCT is an emerging concept in the Western world, and it is expected to herald the next big change in the history of clinical research by bringing about a paradigm shift in the overall shape and scope of RCTs and patient care.

CONCLUSIONS:

PCCTs aim to have increased patient involvement, and are the future of RCTs.

Problems with Randomized Controlled Trials

- Randomized controlled trials (RCTs) are considered to be the gold standard for a clinical trial
- RCTs Form the basis of:
  - Systematic reviews
  - Meta-analyses
  - Treatment guidelines
- The best quality evidence comes from (or is derived from) RCTs

Quality of Evidence in EBM

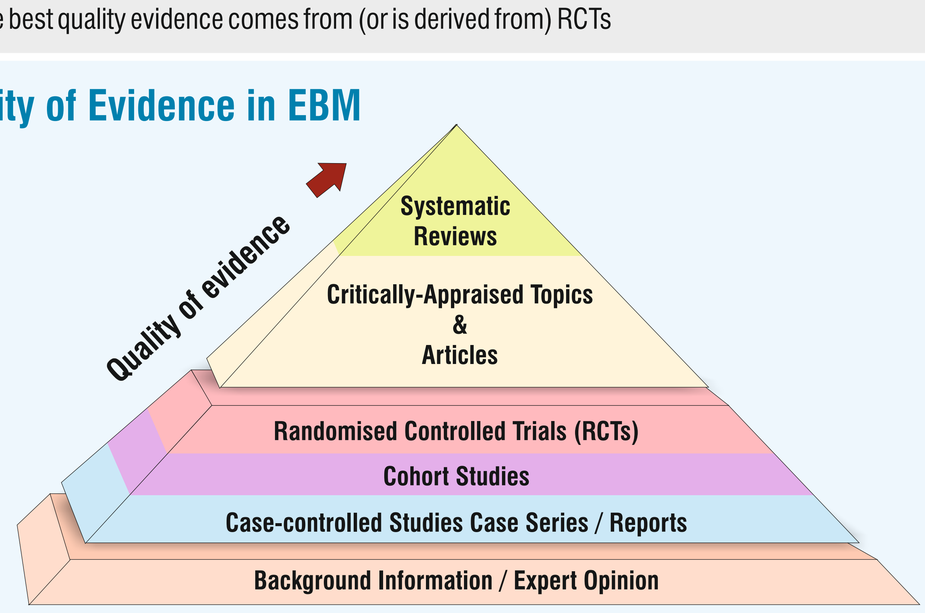

Image source: http://library.uams.edu/help-guides/evidence-based-medicine/evidence-based-medicine-acquiring-evidence-2/

Patient involvement in RCTs

- The ultimate goal of any RCT is the betterment of the health of the patient
- But it is often the case that patients are not involved in the designing of RCTs
- Most of the outcome parameters in RCTs are objective, and very few of them collect patient-centered outcome measures

PCOR: Patient-centered Outcome Research[1]

- The current model for defining effectiveness for a particular treatment modality:
  - Analyzing predominantly 'disease-centered' data
  - This data is almost always 'doctor-reported'
- Optimum and more appropriate model:
  - Analyzing the 'Patient-centered' data as well
  - This subjective data should be ideally 'patient-reported'
- Such an analysis is the core of the term Patient-Centered Outcome Research (PCOR)
- Many industries have routinely started to include patient-reported outcomes (PROs) in their clinical trials to validate the claims of their pharmaceutical products
- A 2004 study found out that PROs were included as efficacy endpoints in approximately 30% of all labels that were reviewed by the USFDA between 1997 and 2002[2]
- The regulators such as the USFDA are increasingly seeking PROs in RCT data for substantiating label claims[3]

Patient involvement in RCTs with PROs

- Even though PROs are included, the extent of patient involvement in RCTs is inadequate, given the fact that the ultimate beneficiaries of RCTs are patients
- In a typical RCT, patients come into picture only after the trial is started
- Even though PROs are included in the protocol, patients themselves do not have a voice in selecting which outcomes are to be included
- After RCT is completed, patients are hardly provided any information about its outcome.

Patient-Centric Clinical Trials (PCCT)

- The concept of Patient-Centric Clinical Trials (PCCT) includes PCOR, and goes a step further
- Patient participation in the RCTs is enhanced
- Patients are involved from the stage of protocol preparation
- RCT data is made easily available to the participating patients

PCCT: Definition[4]

- Patient centricity is a dynamic process through which the patient regulates the flow of information through multiple pathways to exercise choices consistent with his/her preferences, values and beliefs.
- [It entails] more than just the patient's voice; it involves the patient's thoughts, values, preferences, strengths and shortcomings

How is PCCT model different from established trial model?[5]

Established Trial Model	Patient-Centered Clinical Trial
Linear, sequential	Multi-directional, interactive
Compartmentalized	Open
Insular	Integrated
Vertical ownership and centralized risk	Horizontal ownership and shared risk
Rigid, transactional, reactive	Flexible, adaptive, proactive
Proprietary clinical data at the core	Patient experience at core
Focus on great science	Focus on great and feasible science
Participant as study subject	Participant as partner, lead customer

Role of PCORI[6]

- The PCORI (Patient-centered outcomes research institute) in the US has come up with concepts such as:
  - Patient-powered research networks (PPRNs): these are groups of patients interested in forming a research network and in participating in research, both observational and randomized. PPRNs are intended to further explore the role of patient involvement in research topic selection, research recruitment, participation, etc
  - Clinical data research networks (CDRNs): these are networks originating in hospitals which stimulate broader participation of patients, clinicians and payers in performing clinical trials
- PCORI envisions that over time, PPRNs and CDRNs become more integrated and contribute to increased patient involvement in clinical trials

PCCT: Present status and Future

- PCCT is an emerging concept in the Western world
- PCCT is expected to herald the next big change in the history of clinical research by bringing about a paradigm shift in the overall shape and scope of RCTs and patient care.
- Even in India, the concept of PCCT is new
- There are barriers in implementing PCCT in India, because patient involvement in therapy is generally not favoured by Indian practitioners who like to have the full authority in the management

Conclusion

- RCTs have minimal patient involvement
- PCOR and PROs include patient-centered outcome measures, but still fall short because patients don't have a role in selecting outcome measures or designing protocols
- This gap is to be bridged by PCCT
- PCCT is a new concept which aims to have increased patient involvement, right from protocol designing stage

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